



Sarepta Therapeutics Announces Third Quarter 2014 Financial Results and Recent Corporate Developments

- Eteplirsen confirmatory study in ambulatory patients and study in advanced/non-ambulatory patients to begin dosing this month-*
- New Drug Application submission for eteplirsen planned for mid-year 2015 based on recent FDA guidance-*
- Revised 2014 non-GAAP operating loss guidance lowered to \$110-\$120 million with cash and other investments of \$240 million at quarter end-*

CAMBRIDGE, MA, November 6, 2014 -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today reported financial results for the three and nine months ended September 30, 2014, and provided an update of recent corporate developments.

"Our top priority is working as rapidly as possible toward a potential eteplirsen approval, by pursuing a dataset that will satisfy the requirements for an FDA filing and that will stand up to the rigor of the review process," said Chris Garabedian, President and Chief Executive Officer of Sarepta. "We will soon begin dosing with eteplirsen in our confirmatory study in ambulant patients and our study in more progressed and non-ambulant patients, and we are quickly moving forward with additional studies across our DMD program."

Financial Results

For the third quarter of 2014, Sarepta reported a non-GAAP net loss of \$28.8 million, or \$0.70 per share, compared to a non-GAAP net loss of \$21.3 million for the third quarter of 2013, or \$0.63 per share. The incremental loss of \$7.5 million was primarily the result of increased general and administrative expenses as a result of corporate growth as well as a decrease in revenue from the Company's government contracts.

On a GAAP basis, the net loss for the third quarter of 2014 was \$29.2 million, or \$0.71 per share (including \$4.6 million of stock-based compensation expenses), compared to a net loss of \$42.0 million, or \$1.24 per share (including \$3.5 million of stock-based compensation and restructuring expenses) for the third quarter of 2013. The decrease in net loss is primarily due to a favorable change of \$21.4 million in gain (loss) on change in warrant valuation offset by an increase of \$5.6 million in operating expenses and a decrease of \$3.1 million in contract revenue.

Revenue for the third quarter of 2014 was \$1.1 million, down from \$4.2 million for the third quarter of 2013. The \$3.1 million decrease was primarily due to decreases in revenue from the Company's government contracts. The government contract under which the Marburg drug



candidate was being developed expired in July 2014 and the Company is currently evaluating options to continue advancing its Marburg drug candidate and other infectious disease research and development efforts.

Non-GAAP research and development expenses were \$20.2 million for the third quarter of 2014, compared to \$19.9 million for the third quarter of 2013, an increase of \$0.3 million. GAAP research and development expenses were \$21.9 million for the third quarter of 2014 (including \$1.7 million of stock-based compensation expense), compared to \$21.1 million for the third quarter of 2013 (including \$1.2 million of stock-based compensation and restructuring expenses), an increase of \$0.8 million.

Non-GAAP general and administrative expenses were \$9.9 million for the third quarter of 2014, compared to \$5.7 million for the third quarter of 2013, an increase of \$4.2 million. GAAP general and administrative expenses were \$12.9 million for the third quarter of 2014 (including \$3.0 million of stock-based compensation expense), compared to \$8.0 million for the third quarter of 2013 (including \$2.3 million of stock-based compensation expense), an increase of \$4.9 million.

The increase in operating expenses was primarily caused by corporate growth and increased professional fees in the normal course of business.

The Company had \$240.7 million in cash, cash equivalents, short-term investments and restricted investments related to letters of credit as of September 30, 2014 compared to \$264.9 million as of December 31, 2013, a decrease of \$24.2 million. The decrease was primarily driven by the use of cash to fund the Company's ongoing operations in the first nine months of 2014, including the purchase of the Company's manufacturing facility in Andover, offset by the net proceeds received from the Company's public offering in April 2014.

In addition to the GAAP financial measures set forth in this press release, the Company has included certain non-GAAP measurements: non-GAAP research and development expenses, non-GAAP general and administrative expenses, non-GAAP operating expenses, non-GAAP net loss, and non-GAAP basic and diluted net loss per share, which present operating results on a basis adjusted for certain items. The Company uses these non-GAAP measures as key performance measures for the purpose of evaluating performance internally. The Company also believes these non-GAAP measures provide the Company's investors with useful information regarding the Company's historical operating results. These non-GAAP measures are not intended to replace the presentation of the Company's financial results in accordance with GAAP. Use of the terms non-GAAP research and development expenses, non-GAAP general and administrative expenses, non-GAAP operating expenses, non-GAAP net loss, and non-GAAP basic and diluted net loss per share may differ from similar measures reported by other companies. All relevant non-GAAP measures are reconciled from their respective GAAP measures in the attached table "Reconciliation of GAAP to Non-GAAP Net Loss."



2014 Guidance

In light of the recent FDA guidance for the eteplirsen NDA, the Company has reduced planned expenditures for the remainder of the year, and now expects non-GAAP loss from operations to range from \$110 to \$120 million, down from previous guidance of \$135 to \$145 million. The Company expects that its current financial position and prudent expense management will support the execution of its strategic business plan.

The Company cannot provide a reconciliation of this non-GAAP guidance to its relevant GAAP measure because full year loss from operations could include incremental expense related to stock compensation expense.

Recent Corporate Developments

Duchenne Muscular Dystrophy Program

-- Announced updated guidance received from FDA with additional requirements for NDA submission, including the results from an independent assessment of dystrophin images and the 168-week clinical data from study 202, and requests for more specific data including a minimum duration of safety in new patients exposed to eteplirsen, patient-level natural history data to be obtained by Sarepta from independent academic institutions, and MRI data from a recent study conducted by an independent academic group. Based on these requests, the Company plans to submit an NDA by mid-year 2015, pending any additional requests from further discussions with the FDA.

-- Presented data from the ongoing Phase II and preclinical trials evaluating exon-skipping therapies for the treatment of Duchenne muscular dystrophy (DMD) at the International Congress of the World Muscle Society held from October 7-11, 2014, in Berlin, Germany, including an oral presentation with additional detail on the 144-week clinical data, 6-minute walk test (6MWT) and safety data from the Phase IIb study of eteplirsen, along with 144-week pulmonary function test results and information on individual MIP and MEP results.

-- Entered into a multi-year, multi-product partnership with Flagship Biosciences seeking to digitally automate and speed the measurement of dystrophin, a key therapeutic efficacy marker for muscular dystrophy, while ensuring consistency.

Infectious Diseases Program

-- Announced favorable safety results from the single ascending dose portion of a Phase I clinical study of influenza drug candidate AVI-7100, the Company's lead candidate for the treatment of influenza virus, in healthy volunteers. Results showed that AVI-7100 was well-



tolerated up to the highest dose tested of 8 mg/kg, with no reported serious or clinically significant adverse events. An independent Data and Safety Monitoring Board recommended the study continue as planned to the multiple dose portion of the study.

-- Announced the publication of Ebola and Marburg Phase I clinical study results in *Antimicrobial Agents and Chemotherapy*, demonstrating no clinical or toxicologic safety concerns with the Company's drug candidates for the treatment of Ebola and Marburg virus, respectively.

-- Collaborated with the Wellcome Trust, World Health Organization (WHO), and other international authorities in an important program funding clinical trials in West Africa for several experimental Ebola drugs, designed to accelerate the identification and delivery of potential life-saving treatments to those infected by the Ebola virus.

Conference Call

The Company will be hosting a conference call at 8:00 a.m. EST, to discuss these financial results and other corporate updates. The conference call may be accessed by dialing 800-708-4539 for domestic callers and 847-619-6396 for international callers. The passcode for the call is 38322454. Please specify to the operator that you would like to join the "Sarepta Third Quarter 2014 Earnings Call." The conference call will be webcast live under the investor relations section of Sarepta's website at www.sarepta.com and will be archived there following the call for 90 days. Please connect to Sarepta's website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary. An audio replay will be accessible through November 19, 2014 by calling 888-843-7419 or 630-652-3042 and entering access code 38322454#.

About Sarepta Therapeutics

Sarepta Therapeutics is focused on developing first-in-class RNA-based therapeutics to improve and save the lives of people affected by serious and life-threatening rare and infectious diseases. The Company's diverse pipeline includes its lead program eteplirsen, for Duchenne muscular dystrophy, as well as potential treatments for some of the world's most lethal infectious disease. Sarepta aims to build a leading, independent biotech company dedicated to translating its RNA-based science into transformational therapeutics for patients who face significant unmet medical needs. For more information, please visit us at www.sarepta.com.

Forward Looking Statements

In order to provide Sarepta's investors with an understanding of its current results and future prospects, this press release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be



forward-looking statements. Words such as “believes,” “anticipates,” “plans,” “expects,” “will,” “intends,” “potential,” “possible” and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to Sarepta’s future operations, financial performance, business plans, priorities and development of product candidates including: the expected timelines for starting and dosing in clinical studies; the content and planned submission of a New Drug Application (NDA) for eteplirsen and potential approval of that NDA; and our projected Non-GAAP loss from operations.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta’s control. Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include, among others, risks specific to obtaining FDA approval for eteplirsen including: we may not be able to comply with all FDA requests in a timely manner or at all; the FDA may determine that our NDA submission for eteplirsen is incomplete or does not qualify for filing, even if we provide additional supporting information and data; we may not be able to complete clinical trials the FDA requires for approval of eteplirsen and the results of our ongoing and new clinical trials may not be positive or consistent with prior results including possible failure to obtain results supporting safety and efficacy of eteplirsen, our other product candidates and/or Sarepta’s anti-sense based technology platform; there may be delays in our projected timelines relating to eteplirsen clinical studies, our planned NDA submission for eteplirsen, initiating new clinical trials for eteplirsen or other product candidates in our pipeline, or making a product commercially available for various reasons including possible limitations of Company resources and regulatory or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to the patents that cover our product candidate; scale-up of manufacturing may not be successful and any or all of the Company’s drug candidates may fail in development or may not receive required regulatory approvals for commercialization (including potentially under an accelerated pathway); we may need and may not be able to obtain additional funds to conduct our planned research and development efforts and execute our business plans; and those risks identified under the heading “Risk Factors” in Sarepta’s Annual Report on Form 10-Q for the quarter ended September 30, 2014 filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect the Company’s business, results of operations and the trading price of Sarepta’s common stock. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

Internet Posting of Information



We routinely post information that may be important to investors in the 'For Investors' section of our web site at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.



Sarepta Therapeutics, Inc.
Condensed Consolidated Statements of Operations
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Revenues from grants and research contracts	\$ 1,059	\$ 4,168	\$ 9,730	\$ 11,593
Operating expenses:				
Research and development	21,852	21,087	63,399	47,833
General and administrative	12,882	8,014	35,398	21,195
Operating loss	(33,675)	(24,933)	(89,067)	(57,435)
Other income (loss):				
Interest income and other, net	193	63	473	281
Gain (loss) on change in warrant valuation	4,256	(17,160)	(2,779)	(46,011)
Net loss	<u>\$ (29,226)</u>	<u>\$ (42,030)</u>	<u>\$ (91,373)</u>	<u>\$ (103,165)</u>
Net loss per share – basic and diluted	<u>\$ (0.71)</u>	<u>\$ (1.24)</u>	<u>\$ (2.31)</u>	<u>\$ (3.17)</u>
Shares used in per share calculations – basic and diluted	41,066	33,943	39,595	32,588



Sarepta Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Net Loss
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Net loss – GAAP	\$ (29,226)	\$ (42,030)	\$ (91,373)	\$ (103,165)
Research and development:				
Stock-based compensation expense	1,668	1,155	5,886	2,409
Restructuring expense	-	54	11	397
Total research and development non-GAAP adjustments ¹	1,668	1,209	5,897	2,806
General and administrative:				
Stock-based compensation expense	2,981	2,332	8,692	5,067
Restructuring expense	-	-	-	329
Total general and administrative non-GAAP adjustments ¹	2,981	2,332	8,692	5,396
Other non-operating loss:				
(Gain) loss on change in warrant valuation non-GAAP adjustment	(4,256)	17,160	2,779	46,011
Net loss – non-GAAP	<u>\$ (28,833)</u>	<u>\$ (21,329)</u>	<u>\$ (74,005)</u>	<u>\$ (48,952)</u>
Non-GAAP net loss per share – basic and diluted	<u>\$ (0.70)</u>	<u>\$ (0.63)</u>	<u>\$ (1.87)</u>	<u>\$ (1.50)</u>
Shares used in per share calculations – basic and diluted	41,066	33,943	39,595	32,588

¹ Non-GAAP operating expense adjustments are comprised of general and administrative non-GAAP adjustments and total research and development non-GAAP adjustments. Total non-GAAP operating expense adjustments were \$4,649 and \$3,541 for the three months ended September 30, 2014 and 2013, respectively. Total non-GAAP operating expense adjustments were \$14,589 and \$8,202 for the nine months ended September 30, 2014 and 2013, respectively.



Sarepta Therapeutics, Inc.

Balance Sheet Highlights

(in thousands)

(unaudited)

	<i>September 30,</i> <i>2014</i>	<i>December 31,</i> <i>2013</i>
Cash, cash equivalents and short-term investments	\$ 236,017	\$ 256,965
Restricted investments	4,647	7,897
Total assets	319,058	291,569
Total liabilities	32,854	44,377
Total stockholders' equity	\$ 286,204	\$ 247,192

Sarepta Investor Contact:
Stephanie Ascher, 212 362 1200
stephanie@sternir.com

or

Sarepta Media Contact:
Tony Plohoros, 908 591 2839
tplohoros@6degreespr.com